National Institutes of Health Management

House Appropriations Subcommittee on Labor/HHS/Education

**Elias A. Zerhouni, M.D.
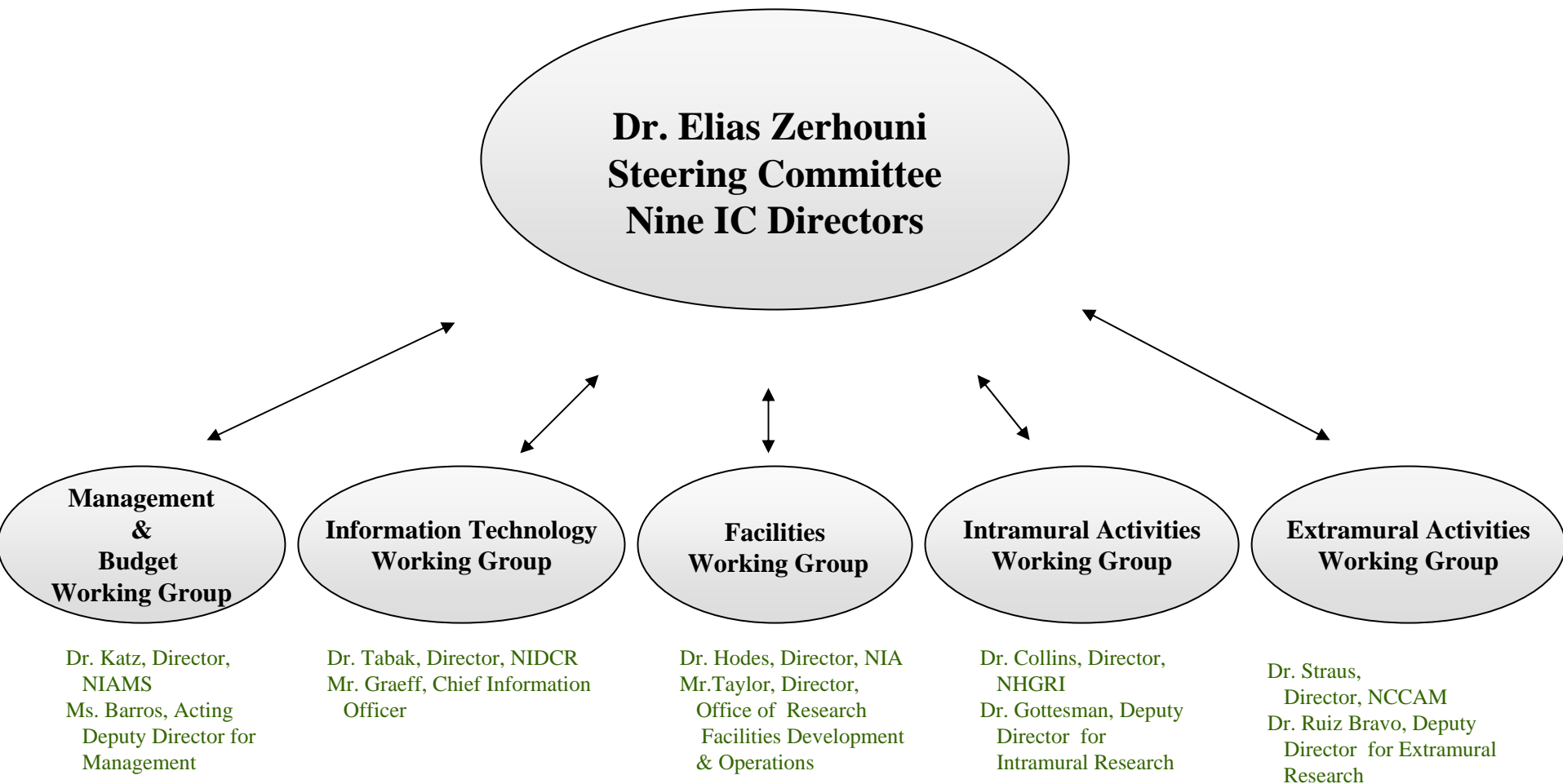Director, National Institutes of Health**

Allen M. Spiegel, M.D., Director, National Institute of Diabetes and Digestive and Kidney Diseases

Story C. Landis, Ph.D., Director, National Institute of Neurological Disorders and Stroke

April 22, 2004

New NIH Decision-making Process

Dr. Elias Zerhouni
Steering Committee
Nine IC Directors

Management & Budget Working Group

Dr. Katz, Director, NIAMS
Ms. Barros, Acting Deputy Director for Management

Information Technology Working Group

Dr. Tabak, Director, NIDCR
Mr. Graeff, Chief Information Officer

Facilities Working Group

Dr. Hodes, Director, NIA
Mr.Taylor, Director, Office of Research Facilities Development & Operations

Intramural Activities Working Group

Dr. Collins, Director, NHGRI
Dr. Gottesman, Deputy Director for Intramural Research

Extramural Activities Working Group

Dr. Straus, Director, NCCAM
Dr. Ruiz Bravo, Deputy Director for Extramural Research

Innovations in NIH Management

- **Administrative Restructurings Underway:**

  Acquisitions, Finance, Budget, Grants Management, Facilities, Equal Employment Opportunity, Information Technology, Human Resources

- **Enterprise Systems Being Implemented:**

  Grants Applications and Awards Management (eRA), Finance and Business Support (NBS), Human Resources (EHRP), Clinical Research Information (CRIS)

- **Future Directions:**

  Data Mining for Disease Funding Tracking
  Leadership Development for Scientific and Administrative Positions

MANAGEMENT EXCELLENCE AND STEWARDSHIP
% Increases in Funding and FTEs over FY 1996 Levels

RESEARCH AND MANAGEMENT EXPENSES DECREASED FROM 4.0% TO 3.5% of NIH BUDGET
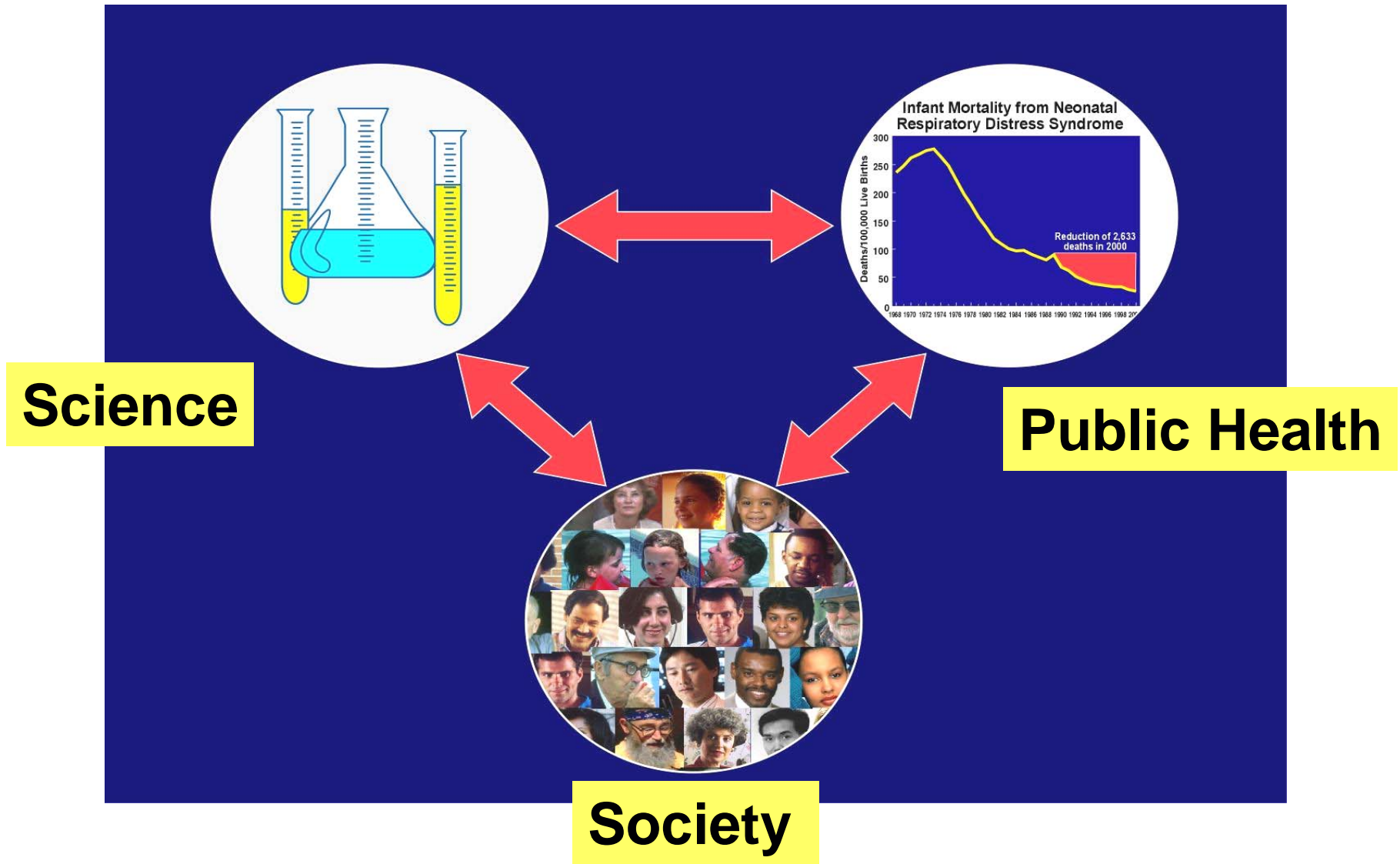
Funding +141%

FTEs +16%
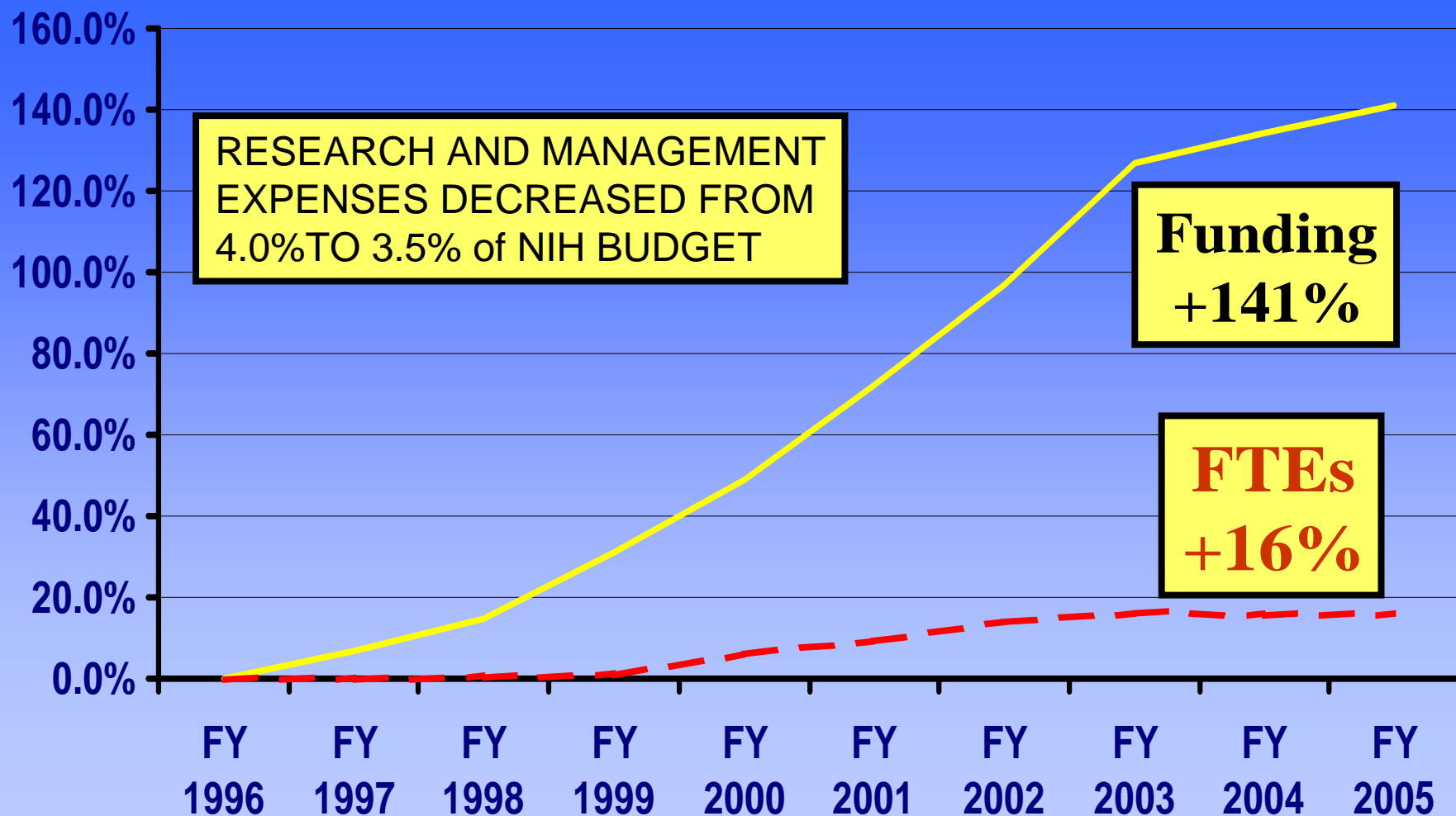
Completion of the Mark O. Hatfield Clinical Research Center

- Largest hospital in the world totally dedicated to clinical research

- Center for the largest number of patients with orphan (rare) diseases in the world

- Provides working environment with unparalleled confluence of basic and clinical researchers and with "Bench to Bedside" literally a few steps down the hall

- Nation's major training center for clinical investigators

- Occupancy – Fall 2004

Effective Portfolio Management

Science

Public Health

Society

Infant Mortality from Neonatal Respiratory Distress Syndrome

Reduction of 2,633 deaths in 2000

NIH Portfolio Management and Priority Setting

Factors in Resource Allocation Decisions

Examples:

- Public Health Needs
- Scientific Opportunities
- Scientific Merit
- Stakeholder Input
  – *patient groups, research community*

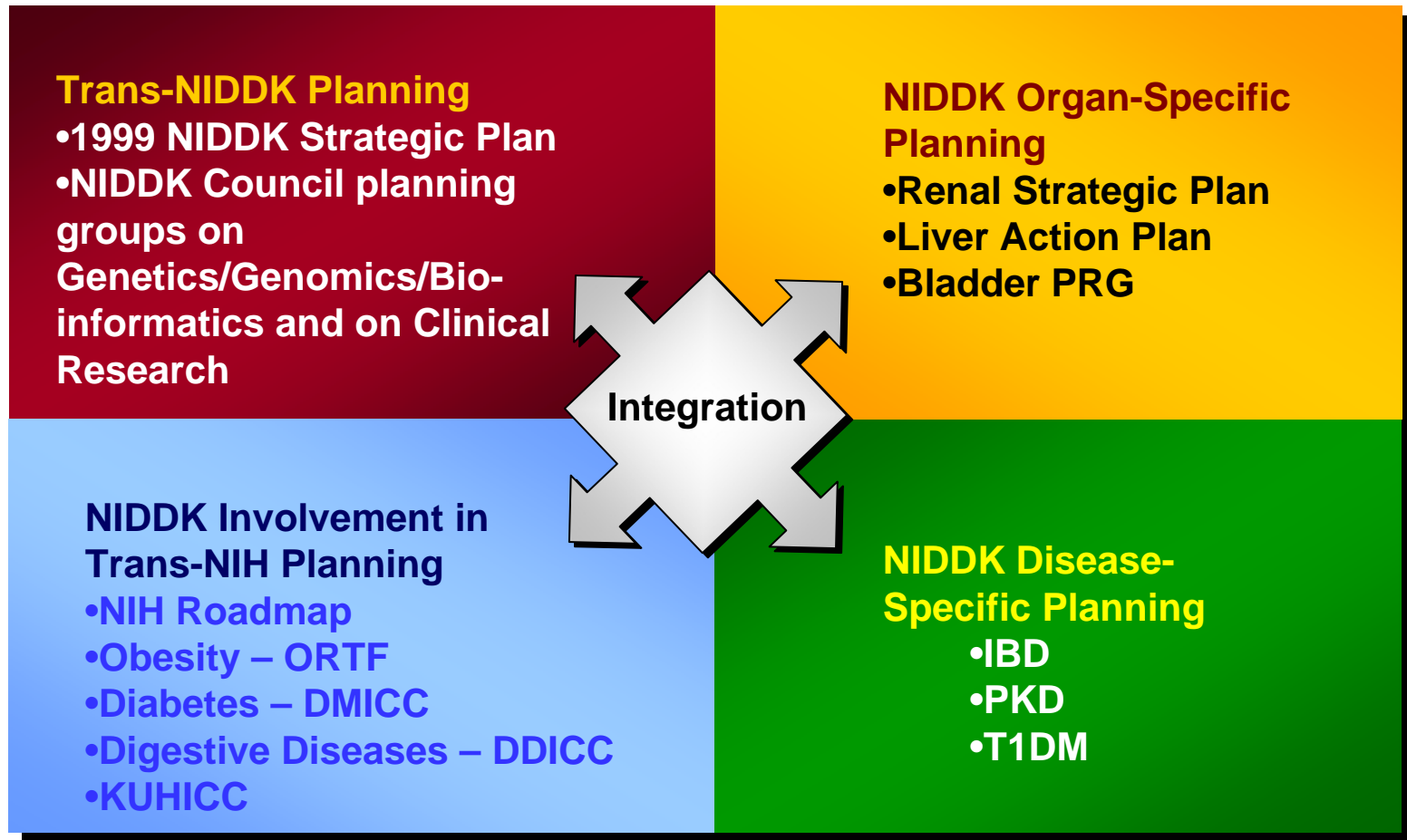# Portfolio Management, cont.

Proactive Management Steps

Examples:

- Strategic and Operational Planning
- Scientific Conferences, Workshops
  - *multi-IC; multidisciplinary*
- Research Solicitations
  - *RFAs, PAs, Contracts*
- New Mechanisms for Supporting Synergistic Research
  - *Consortia, Networks*
- Peer-Review
  - *Study Section and Nat'l Advisory Council*
- Ongoing Evaluation
  - *scientific pubs.; disease trends*

Strategic Planning Efforts To Guide NIDDK Priority Setting

Examples
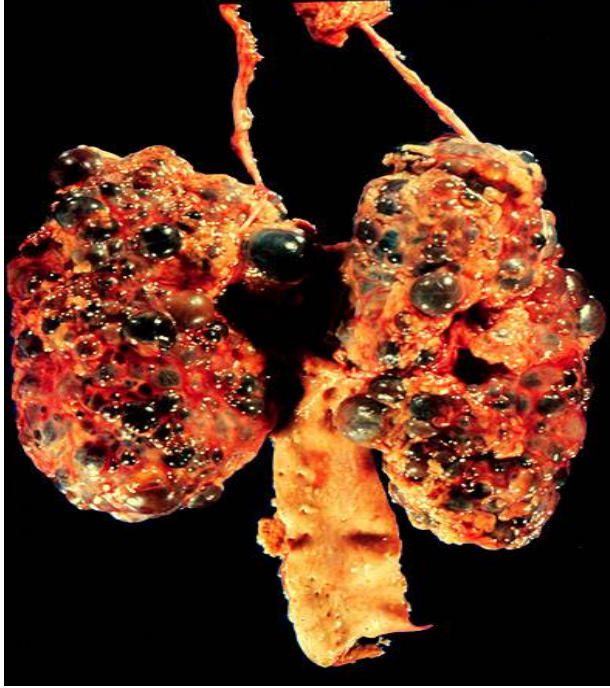
Polycystic Kidney Disease (PKD)

Portfolio Management

Renal Disease Research Plan
PKD Centers
PKD Imaging Consortium

Meeting: Strategic Planning for
Polycystic Kidney Disease

HALT-PKD trial

1994	1995	1996	1997	1998	1999	2000	2001	2002	2003	2004

PKD1 and PKD2 genes found

PKD proteins' function
And location

Candidate Rx for
PKD

Scientific Discoveries

Obesity

Strategic Plan for
NIH Obesity Research

A Report of the NIH Obesity Research Task Force

Portfolio Management

Obesity/Nutrition Research Centers established (92)

Draft Strategic Plan for NIH Obesity Research posted for comments
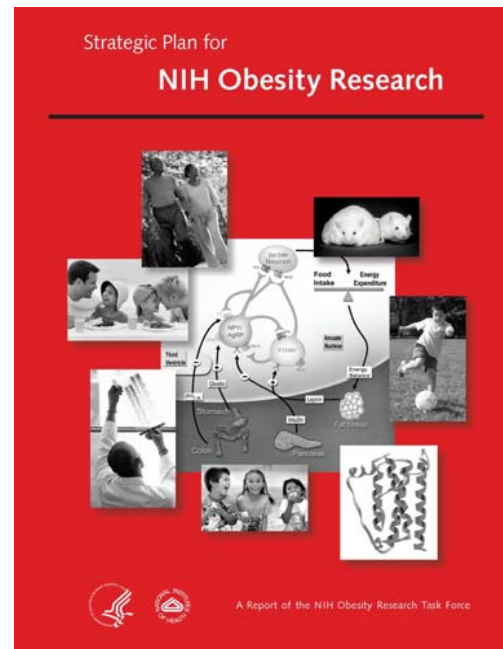
NIH Obesity Research Task Force established

1994	1995	1996	1997	1998	1999	2000	2001	2002	2003	2004

Leptin gene discovered

Ghrelin discovered

Single gene mutations can cause human obesity

Leptin influences brain "wiring"

Scientific Discoveries

Consortium of Neuroscience Institutes
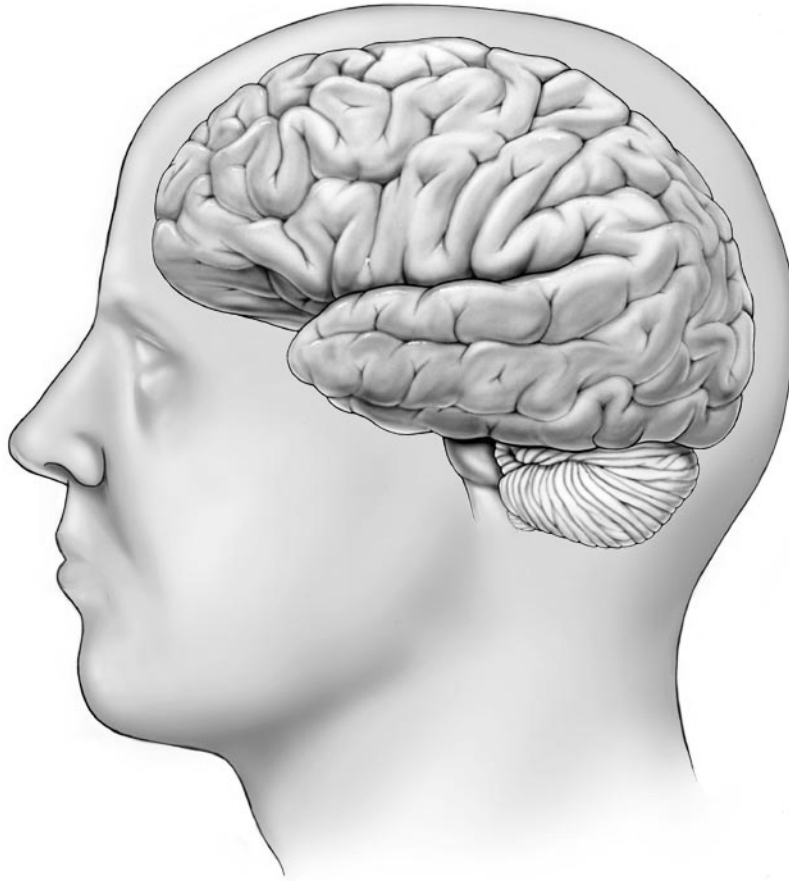
- NINDS
- NIMH
- NIDCD
- NEI
- NIDA
- NIAAA
- NIA
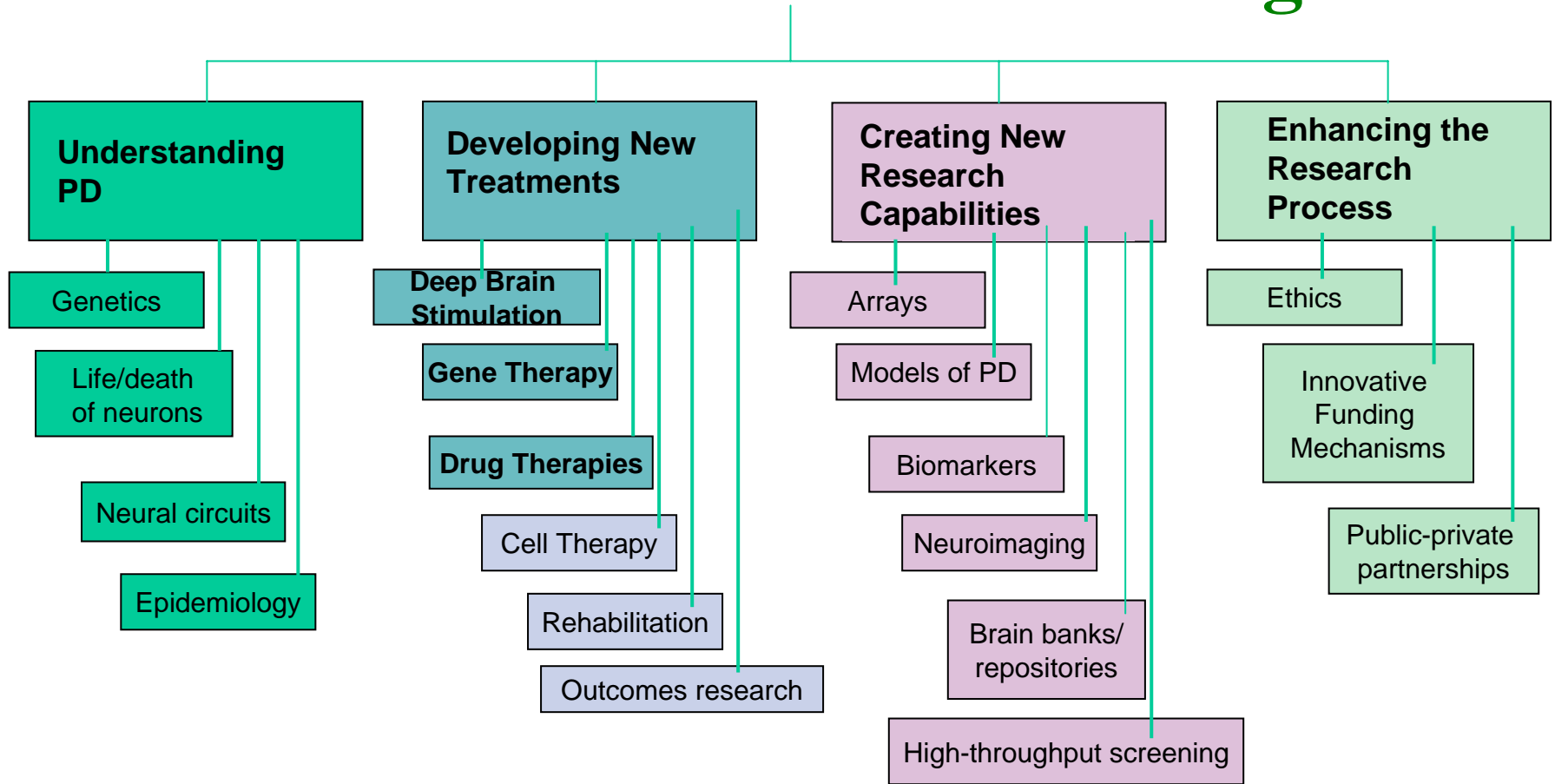- NIDCR
- NICHD

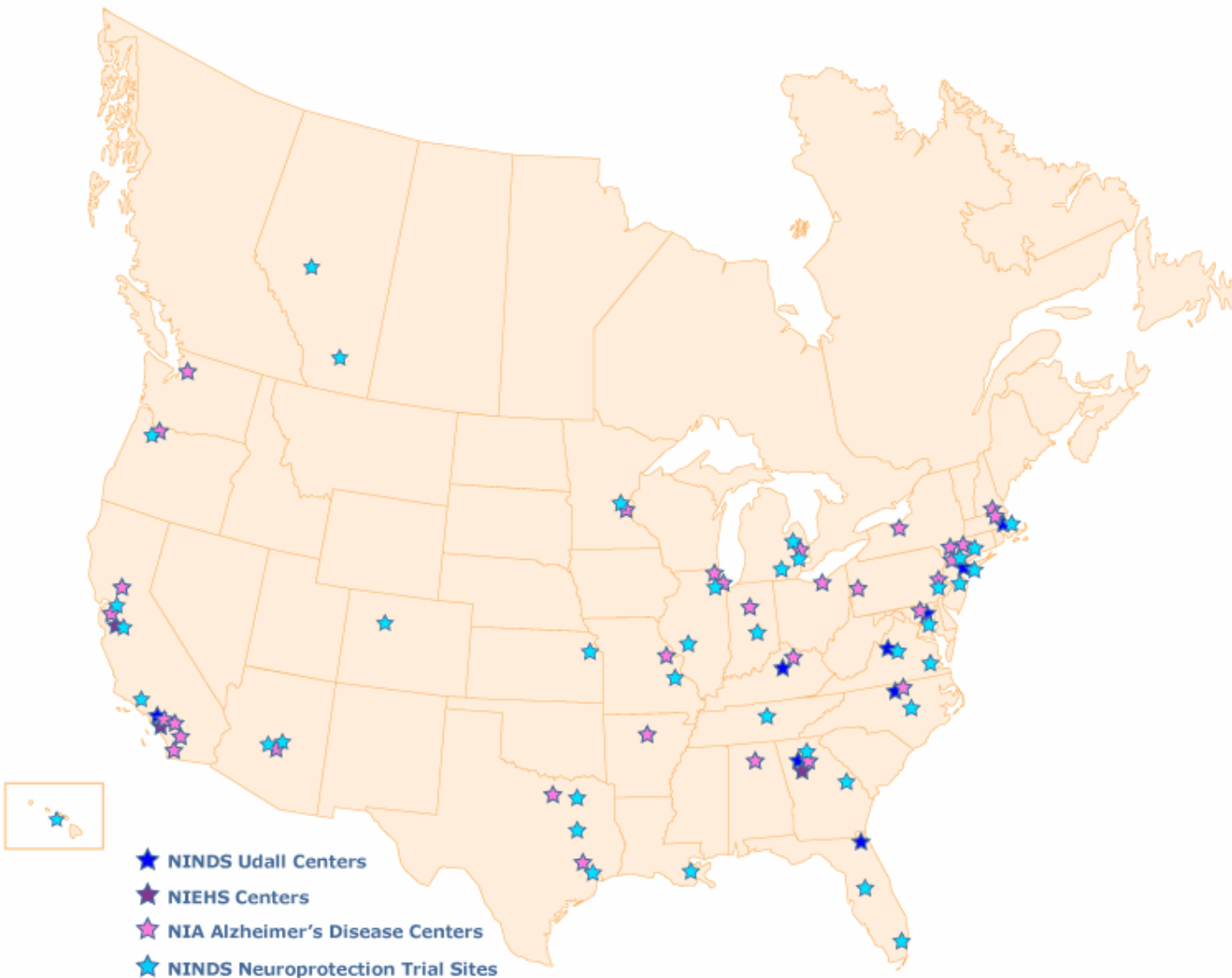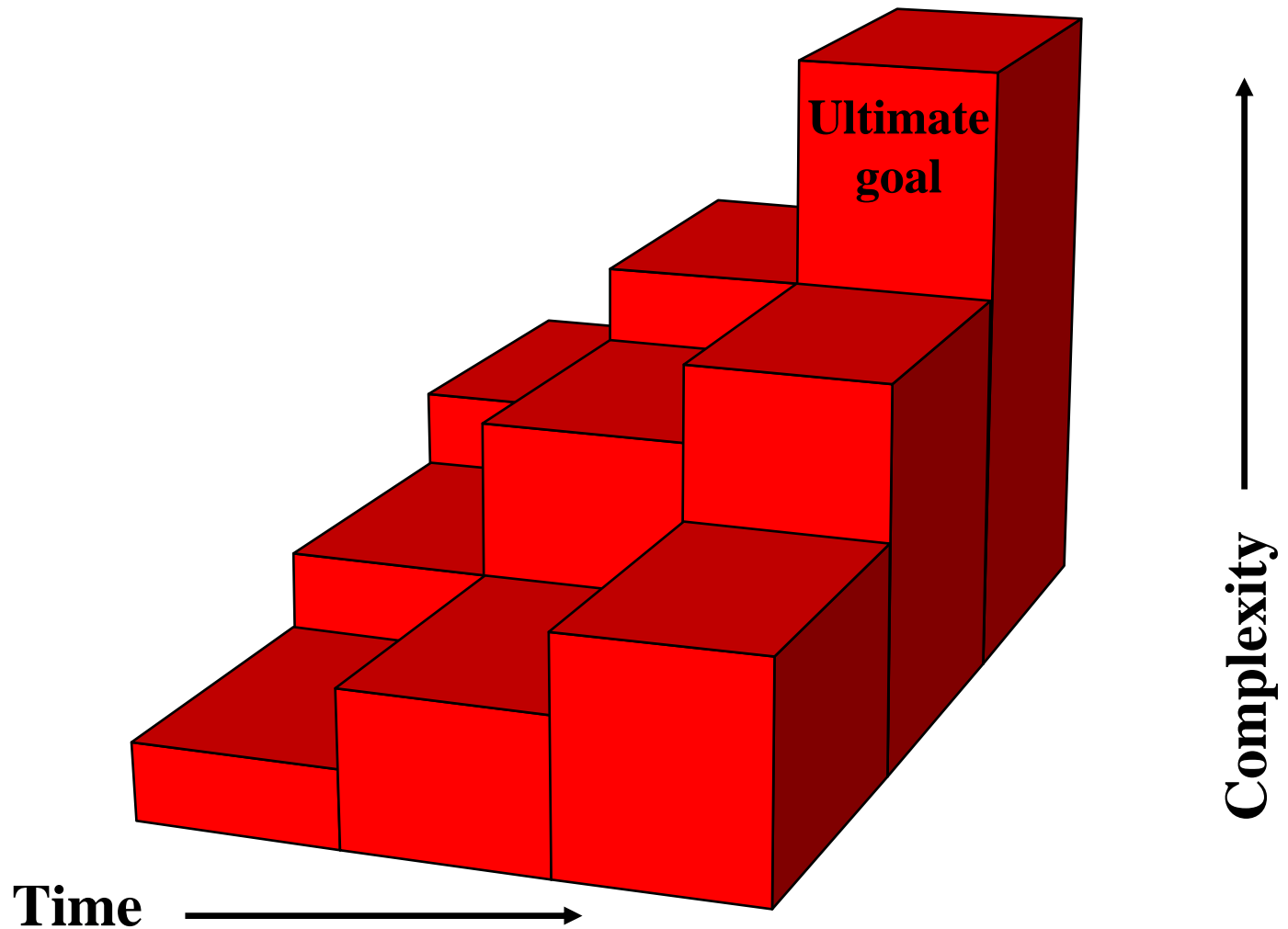**BLUEPRINT FOR THE BRAIN:**

Tools, Technologies, and Training

Parkinson's Disease Research Agenda

Understanding PD

- Genetics
- Life/death of neurons
- Neural circuits
- Epidemiology

Developing New Treatments

- **Deep Brain Stimulation**
- **Gene Therapy**
- **Drug Therapies**
- Cell Therapy
- Rehabilitation
- Outcomes research

Creating New Research Capabilities

- Arrays
- Models of PD
- Biomarkers
- Neuroimaging
- Brain banks/ repositories
- High-throughput screening

Enhancing the Research Process

- Ethics
- Innovative Funding Mechanisms
- Public-private partnerships

NIH CENTERS and NET-PD SITES

★ NINDS Udall Centers
★ NIEHS Centers
★ NIA Alzheimer's Disease Centers
★ NINDS Neuroprotection Trial Sites

NIH Obesity Research Matrix

High risk	**0 – 3 yrs**	**4 – 6 yrs**	**7 – 10 yrs**
	● Identify novel biomarkers that are associated with obesity related co-morbidities using genomic or proteomic approaches across diverse racial and ethnic groups ● Improve public awareness of the effects of overweight on disease risk and quality of life (Public Outreach) ● Use model organisms to identify novel pathways or molecules involved in regulation of body composition, food consumption, or physical activity levels	●●● In well characterized clinical trial populations, test the of novel biomarkers to predict therapeutic efficacy or the progression of co-morbidities associated with obesity ● Identify the relative contributions of various sociocultural, environmental, and behavioral factors to obesity development in adults and children ● Identify genes associated with increased or decreased risk for obesity in metabolically well characterized populations from diverse racial and ethnic groups ● Develop and test strategies to maintain healthy weight in children and adults through behavior (activity, diet) change which can be applied in a home, school, or workplace environment	● Evaluate the effectiveness and assure translation of strategies to maintain healthy weight in children and adults through behavior (activity, diet) change which can be applied in a home, school, or workplace environment ● Use knowledge of regulation of energy storage and food intake to develop new therapeutic modalities (including drugs, surgery, and other technologies) to complement lifestyle interventions ● Use knowledge of mechanisms whereby obesity increases risk for co-morbidities to develop potential therapeutic approaches for ameliorating these conditions independent of weight loss
	● Identify potential cost-effective approaches to modifying environments and policies to support increased physical activity, improved dietary practices, and weight control in children and adults ● Partner with other governmental agencies (local, state, national) to translate successful healthy weight control strategies to practice ● Establish periodic surveys of health care providers treating children and adults to evaluate knowledge, attitudes and behaviors related to weight control in clinical practice ● Establish guidelines for collection of data and samples that would encourage the assessment of multiple co-morbidities within diverse populations	●●● Develop improved technologies, including e-technologies, for the assessment of and as intervention tools related to dietary intake, physical activity ● Determine the relationship between inflammatory markers and obesity ●●● Determine the relationship between mental disorders and the development of obesity or ability to achieve and maintain a healthy weight ● Define the brain regulatory pathways that control food intake and energy metabolism and identify the effects of caloric restriction and increased energy expenditure on energy metabolism, brain regulatory pathways, and body composition	● Identify genetic variants that affect risk of development of co-morbidities, and determine the frequency and population risk in diverse populations ●●● Identify aspects of differential fat distribution or longitudinal patterns of weight change that are associated with greatest health risk in diverse populations ●● Determine frequency and population risk of obesities associated with identified genetic variants ●● Elucidate the role of dietary macronutrient and micronutrient composition on energy metabolism, brain regulatory pathways, appetite, and body composition in humans
	●●● Identify areas where the lack of technology or resources are limiting research progress such as tools to measure energy balance in humans ● Determine age, sex, and race/ethnicity specific estimates of physical activity, dietary intake, and body composition in the US population ● Incorporate the evaluation of public comprehension of public health recommendations in the areas of physical activity, diet and weight control as well as assessment of current weight control/weight loss practices into national and regional health surveys	● Determine the long-term effects of exercise on body composition and obesity-associated health risk independent of weight change ● Partner with private and public organizations involved in health care delivery to identify best practices and consider effective policies and approaches to facilitate translation into clinical care and community settings ●● Elucidate the role of dietary macronutrient and micronutrient composition on energy metabolism, brain regulatory pathways, appetite, and body composition in animal models	●● Use weight loss models (e.g., diet, surgery, physical activity) to understand the biological and psychological factors contributing to weight regain in humans ● Use prospective observational studies to identify behavioral and environmental determinants of excessive weight gain and obesity in children and adolescents ● Determine the effects of nutritional/environmental factors at critical periods throughout life such as fetal, neonatal, adolescence, during pregnancy, menopause, and in older adulthood on the central and peripheral nervous system

Low risk

Effective Portfolio Management

Science

Public Health

Society